THE NIH TOOLBOX IN DUCHENNE MUSCULAR DYSTROPHY

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INTRODUCTION: Methodological barriers have hampered continuity of assessment of cognitive function in Duchenne muscular dystrophy (DMD), a multi-organ systemic disease. This barrier has not only affected recommendations regarding clinical care guidelines, but has prevented the acceleration of preclinical successes into human clinical studies.

OBJECTIVE: To systemically investigate the validity of the NIH Toolbox Cognition Battery to assess the cognitive profile in DMD. The NIH Toolbox is a psychometrically sound neuro-behavioral measure that allows for continuity of measurement across the lifespan. It can be easily administered via an iPad, and can be completed within 20-35 minutes. Most importantly, it fulfills the NIH's mandate for scientific rigor and reproducibility, and is compliant with the FDA's requirements of data collection and data storage.

METHODS: We administered the NIH Toolbox in 30 boys with DMD.

RESULTS: All participants completed the battery without any interruptions. Our results find that the total cognition score was 1-1.5 standard deviations below age-expected standard scores. Cognitive strength was noted in crystalized cognition score whereas remarkable vulnerability was noted in fluid cognition scores, with scores ranging 1-2 standard deviations below age-expected standard scores. These results are consistent with previous reports of cognitive profile in DMD.

SUMMARY/CONCLUSION: The NIH Toolbox reliably assesses the cognitive profile in DMD. The user-friendly technologically enabled tool not only allows for scalability as in multicenter clinical trials, it can also be easily adopted for routine clinical care. Thus, the NIH Toolbox has both clinical and research applications in DMD.

Disclosures:

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